

## Treatment for Spinal Cord Injury To Start Clinical Trial Funded by California's Stem Cell Agency

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**San Francisco, CA** – Asterias Biotherapeutics Inc. has been given approval by the Food and Drug Administration (FDA) to begin a clinical trial of its stem cell therapy in patients with spinal cord injury. The California Institute for Regenerative Medicine (CIRM), California's stem cell agency, is funding the trial.

The Phase 1/2a clinical trial is designed to test the safety of this approach and to see if it has any benefit for patients. The trial will use increasing doses of Asterias' AST-OPC1 cells. These are a form of cell called oligodendrocyte progenitor, which are capable of becoming several different kinds of cells some of which play a supporting role and help protect nerve cells in the central nervous system, the area damaged in spinal cord injury.

The trial is a follow-on to the CIRM-funded clinical trial begun by Geron in 2010 using the same kind of stem cell. Geron halted that clinical trial due to a change in business strategy. At the time five patients had been treated with the AST-OPC1 cells. Since then follow-up studies on the five patients have shown no serious side effects due to the therapy and in four of the five patients, MRI scans have shown that the actual injury site had shrunk and that the cells may have had some positive effects in reducing the deterioration of spinal cord tissue.

"This is exactly the type of treatment, focusing on an unmet medical need, that CIRM was created to address," says C. Randal Mills, Ph.D., the President and CEO of the stem cell agency. "There are around 1.3 million Americans living with spinal cord injuries and there are currently no effective treatments. These injuries have enormous impact on the quality of the lives of the patients they affect and the lives of those around them. A therapy that can potentially help them is something worth accelerating."

"We would like to acknowledge the scientists, clinical investigators, and FDA for working with us to develop AST-OPC1," says Pedro Lichtinger, President and CEO of Asterias. "We are especially enthusiastic about working with our new partner, CIRM, in executing this clinical trial. The FDA clearance provides Asterias with imminent access to the previously announced \$14.3 million CIRM grant, which provides non-dilutive funding to support both the clinical trial and other product development activities for AST-OPC1."

This new trial will differ from the Geron trial in that it will involve doses of stem cells up to ten times greater than in the initial trial. It will also focus on patients with complete spinal cord injuries originating in the neck rather than the back. The 13 patients to be treated will all have experienced injuries in the C5-C7 vertebrae and will have essentially lost all feeling and movement below the injury site, with severe paralysis of the upper and lower limbs. Patients will be treated with the cells 14 to 30 days after the injury was sustained. Asterias expects to begin patient enrollment in early 2015.

Katie Sharify was one of the patients enrolled in the Geron trial. She says she was disappointed when that trial was halted so she is delighted to see Asterias advancing the research: "A lot remains unknown about human embryonic stem cells and that's exactly why this research is so important. The scientific community is going to have a much greater understanding of these stem cells from the data that will be collected throughout the study and I'm glad to have been a part of this advancement."

Roman Reed, who received a spinal cord injury in a 1994 football accident, said he was delighted that Asterias has been given approval to start a clinical trial. "The announcement of FDA approval for a Phase I/2a clinical trial is an enormous leap forward for the field of paralysis treatment. I am so excited to have helped start this research with the great stem cell pioneer Dr. Hans Keirstead, and even more positive about our prospects for cure!! One day, we will have our cures and this trial gets us that much closer. Carpe Diem!"

### About CIRM

CIRM was established in November 2004 with the passage of Proposition 71, the California Stem Cell Research and Cures Act. The statewide ballot measure, which provided \$3 billion in funding for stem cell research at California universities and research institutions, was overwhelmingly approved by voters, and called for the establishment of an entity to make grants and provide loans for stem cell research, research facilities, and other vital research. For more information go to <http://www.cirm.ca.gov>

### About Asterias Biotherapeutics

Asterias' core technologies center on stem cells capable of becoming all of the cell types in the human body, a property called pluripotency. Asterias plans to develop therapies based on pluripotent stem cells to treat diseases or injuries in a variety of medical fields having major unmet needs and without adequate therapies available. Asterias initial focus is on two clinical stage programs including oligodendrocyte progenitor cells (AST-OPC1) for spinal cord injuries and antigen-presenting allogeneic dendritic cells (AST-VAC2) for lung cancer.

In October of 2013, Asterias acquired the cell therapy assets of Geron Corporation. These assets included INDs for the clinical stage AST-OPC1 and AST-VAC1 programs, banks of cGMP-manufactured AST-OPC1 drug product, cGMP master and working cell banks of human embryonic stem cells, over 400 patents and patent applications filed worldwide including broad issued claims to fundamental platform technologies for the scalable growth of pluripotent stem cells and compositions of matter for several hESC-derived therapeutic cell types, research cell banks, customized reagents and equipment, and various assets relating to the AST-VAC2 program and preclinical programs in cardiology, and orthopedics.

Asterias Series A Common Stock is traded on the OTC Bulletin Board under the symbol ASTY. Additional information about Asterias can be found at [www.asteriasbiotherapeutics.com](http://www.asteriasbiotherapeutics.com)

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